

BETH C. DRAIN, CA CSR NO. 7152

BEFORE THE
SCIENCE SUBCOMMITTEE OF THE
INDEPENDENT CITIZENS' OVERSIGHT COMMITTEE
TO THE
CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE
ORGANIZED PURSUANT TO THE
CALIFORNIA STEM CELL RESEARCH AND CURES ACT
REGULAR MEETING

LOCATION: AS INDICATED ON THE AGENDA

DATE: NOVEMBER 8, 2018
10 A.M.

REPORTER: BETH C. DRAIN, CA CSR 7152

FILE NO.: 2018-15

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I N D E X

ITEM DESCRIPTION	PAGE NO.
OPEN SESSION	
1. CALL TO ORDER.	3
2. ROLL CALL.	3
3. CONSIDERATION OF AMENDMENTS TO THE CONCEPT PLANS FOR TRANSLATION AND CLINICAL STAGE RESEARCH PROGRAMS.	4
4. PUBLIC COMMENT.	NONE
6. ADJOURNMENT.	24

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NOVEMBER 8, 2018; 10 A.M.

CHAIRMAN SHEEHY: I'D LIKE TO CALL THE MEETING OF THE SCIENCE SUBCOMMITTEE TO ORDER NOW. MARIA, COULD YOU CALL THE ROLL PLEASE.

MS. BONNEVILLE: SURE. DEBORAH DEAS.

DR. DEAS: HERE.

MS. BONNEVILLE: ANNE-MARIE DULIEGE.

DR. DULIEGE: YES.

MS. BONNEVILLE: DAVID HIGGINS.

DR. HIGGINS: HERE.

MS. BONNEVILLE: STEVE JUELSGAARD.

MR. JUELSGAARD: PRESENT.

MS. BONNEVILLE: BERT LUBIN. SHLOMO MELMED. JEFF SHEEHY.

CHAIRMAN SHEEHY: HERE.

MS. BONNEVILLE: OS STEWARD.

DR. STEWARD: HERE.

MS. BONNEVILLE: JONATHAN THOMAS.

CHAIRMAN THOMAS: HERE.

MS. BONNEVILLE: ART TORRES. KRISTINA VUORI.

DR. VUORI: HERE.

MS. BONNEVILLE: THANK YOU.

CHAIRMAN SHEEHY: OKAY.

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1 MS. BONNEVILLE: WE HAVE A QUORUM.

2 CHAIRMAN SHEEHY: GREAT. I THINK GIL, DR.
3 SAMBRANO, DO YOU HAVE A PRESENTATION FOR US? YOU
4 GOING TO LEAD US THROUGH THIS?

5 DR. SAMBRANO: YES, I DO, MR. SHEEHY. LET
6 ME START WITH THE SLIDE DECK WE HAVE DISTRIBUTED AND
7 IS AVAILABLE ON WEBEX. SO I'M GOING TO BE GOING
8 THROUGH THAT BY SLIDE. SO IT, HOPEFULLY, SHOULD BE
9 PRETTY STRAIGHTFORWARD.

10 WE'VE BEEN DISCUSSING, AND THIS WILL BE A
11 CONTINUATION OF OUR DISCUSSION, ON THE POSSIBILITY
12 OF EXPANDING ELIGIBILITY OF CIRM PROJECTS TO INCLUDE
13 THOSE THAT HAVE GENE THERAPY APPROACHES. WE
14 DISCUSSED THIS PREVIOUSLY AT A FULL BOARD MEETING,
15 AND A REQUEST WAS MADE TO REALLY HAVE A BETTER
16 DEFINITION OF SCOPE FOR THE TYPES OF PROJECTS THAT
17 WE ARE INTENDING TO BRING INTO SCOPE, AND WITHIN
18 THAT MAYBE INCLUDE A DEFINITION FOR WHAT GENE
19 THERAPY MEANS TO CIRM.

20 THERE WAS ALSO A REQUEST TO BRING PROJECTS
21 THAT ARE GENE THERAPY, BUT ALSO THAT HAVE A FOCUS ON
22 REGENERATIVE MEDICINE AS A COMPONENT OF THAT. SO WE
23 PROVIDED A MEMO ALSO IN THE MATERIALS THAT WE
24 PROVIDED TO YOU THAT PRESENTS A LITTLE BIT OF THE
25 RATIONALE BEHIND OUR INTENT BEHIND THE GENE THERAPY.

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1 SO JUST TO START, I WANT TO MAKE A FEW
2 POINTS. FIRST, THAT GENE THERAPY THAT HAS INVOLVED
3 STEM OR PROGENITOR CELLS IS CURRENTLY ELIGIBLE UNDER
4 EXISTING RULES. SO THAT HAS BEEN THE CASE. WE HAVE
5 FUNDED PROJECTS THAT COMBINE GENE THERAPY WITH STEM
6 OR PROGENITOR CELLS.

7 SO THE PROPOSAL THAT WE ARE DISCUSSING IS
8 ONE THAT WOULD BRING IN GENE THERAPY APPROACHES THAT
9 DO NOT INVOLVE STEM OR PROGENITOR CELLS. SO JUST
10 WANT TO BE CLEAR ABOUT THAT. AND AS A CONSEQUENCE,
11 IT REQUIRES THAT THE GWG OR GRANTS WORKING GROUP,
12 THROUGH A TWO-THIRDS MAJORITY VOTE, DEEM SUCH
13 PROJECTS A VITAL RESEARCH OPPORTUNITY. SO I THINK
14 OUR DISCUSSION AND OUR RATIONALE HERE ALSO WILL
15 CONTRIBUTE TO THE GWG ASSESSMENT OF WHETHER THEY
16 FEEL IT'S A VITAL RESEARCH OPPORTUNITY OR NOT. SO
17 THAT'S JUST TO START OFF WITH.

18 SO WHAT WE'VE DONE, BASED ON PREVIOUS
19 DISCUSSION AND, AGAIN, THE REQUEST TO FOCUS ON
20 REGENERATIVE MEDICINE AND UNMET MEDICAL NEEDS, WE
21 DEFINED THE PROJECT ELIGIBILITY THAT WOULD GO INTO
22 OUR SOLICITATIONS AS SHOWN ON THIS SLIDE.

23 SO THERE ARE THREE BASIC CONDITIONS THAT
24 WOULD NEED TO BE MET. SO THE FIRST ONE IS THAT
25 EITHER IT TARGETS A STEM CELL IN EFFECT WHICH, AS I

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1 ALREADY STATED, IS ALREADY ELIGIBLE, OR IT'S A GENE
2 THERAPY APPROACH THAT TARGETS ANY OTHER SOMATIC CELL
3 IF IT'S DEEMED A VITAL RESEARCH OPPORTUNITY BY THE
4 GRANTS WORKING GROUP.

5 SECONDLY, IT MUST BE INTENDED TO REPLACE,
6 REGENERATE, OR REPAIR THE FUNCTION OF AGED, DISEASE
7 DAMAGED OR DEFECTIVE CELLS, TISSUES, AND/OR ORGANS.
8 SO THAT IS THE REGENERATIVE MEDICINE COMPONENT.

9 AND, FINALLY, THAT IT'S BEING DEVELOPED
10 FOR A RARE, UNMET MEDICAL NEED UNLIKELY TO RECEIVE
11 FUNDING FROM OTHER SOURCES. AND THAT'S TO EMPHASIZE
12 BOTH THE FACT THAT THIS IS FOR MEDICAL PURPOSES AND
13 ALSO TO HELP SUPPORT WHAT WE HAVE USUALLY DONE UNDER
14 PROP 71 TO SUPPORT THOSE PROJECTS THAT ARE UNLIKELY
15 TO RECEIVE FUNDING FROM OTHER SOURCES.

16 ALL RIGHT. IN THE NEXT SLIDE, HOW WE, AT
17 LEAST FOR NOW, HAVE COME DOWN TO DEFINING GENE
18 THERAPY FOR THE PURPOSE OF THE SOLICITATIONS FOR THE
19 TRANSLATIONAL AND CLINICAL PROGRAMS ARE AS FOLLOWS:
20 SO WE WOULD CONSIDER GENE THERAPY TO MEAN A HUMAN
21 THERAPEUTIC INTERVENTION THAT'S INTENDED TO DO
22 EITHER, ONE, TO ALTER THE GENOMIC SEQUENCE OF CELLS.
23 SO THIS CAN BE WHETHER IT'S IN VIVO OR EX VIVO. WE
24 DON'T HAVE ANY PREFERENCE ONE WAY OR THE OTHER, BUT
25 IT MUST ACT ON THE GENOME SEQUENCE.

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1 OR, NO. 2, ALTER THE CELLULAR LINEAGE VIA
2 GENE DELIVERY; I.E., DIRECT LINEAGE REPROGRAMMING.
3 SO THIS CAME ABOUT BECAUSE IN OUR DISCUSSIONS ABOUT
4 THE POTENTIAL BREADTH OF GENE THERAPY, WE THOUGHT
5 THAT IT COULD BE DEFINED QUITE BROADLY TO INCLUDE
6 THINGS THAT ALTER GENE EXPRESSION. WE THOUGHT THAT
7 DIRECT LINEAGE REPROGRAMMING, BECAUSE IT EMANATES IN
8 MANY WAYS FROM STEM CELL RESEARCH, WAS AN IMPORTANT
9 AREA THAT WE WANTED TO INCLUDE.

10 SO BASICALLY WE ARE INCLUDING TWO BASIC
11 AREAS, THOSE THAT ALTER THE GENOME SEQUENCE OR ALTER
12 THE CELLULAR LINEAGE VIA GENE DELIVERY. AND AS
13 INDICATED IN THE SLIDE, THESE TYPES OF INTERVENTIONS
14 CAN INCLUDE STRATEGIES TO REPAIR A GENE SEQUENCE,
15 REMOVE OR INACTIVATE A DISEASE-CAUSING GENE, OR
16 INTRODUCING NEWER, MODIFIED GENES THAT AUGMENT THE
17 POTENTIAL OF THE TARGET CELLS, SUCH AS WITH CAR-T
18 CELL THERAPIES.

19 SO THAT'S KIND OF THE SCOPE AND DEFINITION
20 OF GENE THERAPY AS WE ARE PROPOSING.

21 I ALSO WANT TO TALK A LITTLE BIT ABOUT THE
22 PROCESS THAT WE ARE ENVISIONING TO MOVE THIS FORWARD
23 ASSUMING THAT THIS IS APPROVED. CONVEY TO CONSIDER
24 THIS TYPE OF GENE THERAPY AS WE ARE PROPOSING TO YOU
25 AND AS IT WOULD BE APPROVED, HAVE THE GWG VOTE ON

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1 WHETHER THEY FEEL THAT THESE EFFORTS AS A WHOLE
2 REPRESENT A VITAL RESEARCH OPPORTUNITY TO CIRM. AND
3 ASSUMING THAT THEY HAVE A POSITIVE VOTE ON THAT,
4 WHAT THAT WOULD ALLOW US TO DO IS SIMPLY ACCEPT ALL
5 APPLICATIONS THAT MEET THESE NEW CRITERIA WITHOUT
6 HAVING TO MODIFY OUR PROCESS FOR EACH OF THE
7 APPLICATIONS AS THEY COME IN. SO BASICALLY WE WOULD
8 MANAGE IT UNDER A SINGLE VOTE RATHER THAN HAVING TO
9 TAKE A VOTE EVERY TIME WE HAVE A PROJECT IN WAITING
10 UNTIL THE GWG TO HAVE THAT HAPPEN. WE JUST THINK
11 THIS MAY EXPEDITE THE PROCESS ALTOGETHER.

12 SO THAT CONCLUDES MY PRESENTATION AND
13 HAPPY TO ADDRESS ANY QUESTIONS.

14 CHAIRMAN SHEEHY: SO DO WE HAVE -- ARE
15 THERE ANY QUESTIONS ABOUT THE NEW DEFINITION MAYBE
16 OVERALL?

17 DR. JUELSGAARD: I HAVE QUESTIONS OUTSIDE
18 OF THE DEFINITION.

19 CHAIRMAN SHEEHY: OKAY. GO AHEAD.

20 DR. JUELSGAARD: SO, DR. SAMBRANO, I READ
21 THE MEMO THAT TALKS ABOUT THE REASONS WHY THIS MIGHT
22 BE A GOOD IDEA, BUT WHAT'S MISSING FROM THE MEMO IS
23 THE OTHER SIDE OF THE COIN. SO EVERYTHING IS
24 ALWAYS A MATTER OF PLUSES AND MINUSES, AT LEAST IN
25 MY EXPERIENCE. WHAT DO YOU SEE AS THE POTENTIAL

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1 NEGATIVES FOR ADOPTING THIS PROGRAM OR THIS NEW
2 MODALITY THAT WOULD BE CONSIDERED FOR CIRM FUNDING?

3 DR. SAMBRANO: SURE. I CAN TELL YOU MY
4 PERSONAL OPINION. OTHERS MAY WANT TO WEIGH IN.
5 MARIA MAY WANT TO WEIGH IN ON THIS AS WELL. I SEE A
6 FEW DOWNSIDES. I THINK THAT OUR GOAL IS TO EXPAND
7 TO BROADLY CONSIDERED DIFFERENT REGENERATIVE
8 MEDICINE APPROACHES. THERE IS OBVIOUSLY THE
9 POTENTIAL THAT IF THERE ARE MORE NONSTEM CELL AND
10 GENE-THERAPY-ONLY APPROACHES, THAT THERE IS SOME
11 DILUTION OF FUNDS GOING TO OUR CORE STEM CELL
12 PROJECTS. I THINK THAT WOULD BE, PERHAPS, THE
13 BIGGEST CONSIDERATION; BUT I DON'T KNOW IF ANY
14 OTHERS OR IF ANYBODY ELSE WANTS TO WEIGH IN ON THAT.

15 CHAIRMAN SHEEHY: GO AHEAD, STEVE.

16 DR. JUELSGAARD: I WAS JUST GOING TO SAY
17 THAT'S EXACTLY WHAT I WAS GETTING AT. WE CALL IT A
18 DILUTION OR CALL IT WHATEVER, BUT EVERY TIME WE
19 APPROVE SOMETHING UNDER THIS NEWER DEFINITION,
20 ASSUMING THAT HAPPENS, THAT MONEY GETS DEVOTED TO
21 THAT PARTICULAR USE. AND NOW ANOTHER EQUALLY,
22 POTENTIALLY EQUALLY WORTHY, ALBEIT LATER IN TIME,
23 STEM CELL PROJECT COMES UP, WE WILL NOT HAVE THE
24 MONEY TO FUND IT. IN OTHER WORDS, WE ARE TAKING
25 MONEY THAT WE HAVE HISTORICALLY BEEN DEVOTING TO

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1 OTHER REGENERATIVE MEDICINE THERAPIES AND
2 POTENTIALLY TO USE IT IN A DIFFERENT BUCKET, AND THE
3 CONSEQUENCE COMES WITH A COST. AND THAT COST IS THE
4 MONEY THAT IS NO LONGER USABLE FOR WHERE WE HAVE
5 ALWAYS USED IT. AND SO I THINK IT'S JUST IMPORTANT,
6 AS WE CONSIDER WHETHER OR NOT TO MOVE FORWARD IN
7 THIS DIRECTION, THAT WE NEED TO CONSIDER WHETHER
8 THAT'S SOMETHING WE TRULY WANT TO DO OR NOT.

9 I UNDERSTAND IT'S A BRAND NEW, EXCITING
10 THERAPEUTIC AREA. AND IT'S ONE OF MANY, RIGHT? SO
11 IT DOESN'T SIT BY ITSELF. GENE THERAPY DOESN'T SIT
12 BY ITSELF IN TERMS OF ADVANCES IN SCIENCE. SCIENCE
13 IS ADVANCING ALL THE TIME. FOR ME IT'S MORE A
14 MATTER OF FOCUS AND WHETHER OR NOT WE BEGIN TO
15 DILUTE THE FOCUS THAT WE'VE HAD BY BRINGING IN OTHER
16 POSSIBILITIES. I WOULD NOTE THIS AND I'LL BE DONE
17 HERE.

18 AT THE LAST MEETING WE DECIDED ACTUALLY TO
19 LIMIT THE SCOPE OF OUR REGENERATIVE MEDICINE
20 CONSIDERATIONS BY SAYING ESSENTIALLY THAT WE WEREN'T
21 GOING TO FUND PROJECTS THAT INVOLVE SMALL MOLECULES
22 OR BIOLOGICS UNLESS THEY WERE SOMETHING WE HAD
23 ALREADY HAD IN THE QUEUE WE HAD APPROVED BEFORE. SO
24 ON THE ONE HAND, WE NARROWED OUR REGENERATIVE
25 MEDICINE FOCUS WITH THAT DECISION. ON THE OTHER

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1 HAND, NOW WE ARE CONSIDERING EXPANDING BEYOND
2 REGENERATIVE MEDICINE. AND IT'S JUST A BIT OF A
3 MISMATCH FROM MY POINT OF VIEW.

4 I'M FINISHED, JEFF.

5 CHAIRMAN SHEEHY: DO OTHER MEMBERS HAVE
6 COMMENTS?

7 DR. STEWARD: I HAVE COMMENTS, BUT IT
8 REALLY IS ABOUT THE VOTING PROCESS AND NOT SO MUCH
9 THE PROPOSED MODIFICATIONS PER SE. I WILL SAY I
10 SHARE SOME OF STEVE'S CONCERNS OR AT LEAST I WOULD
11 ALSO AGREE THAT THESE ARE POTENTIAL ISSUES. IF THIS
12 WAS IN THE BEGINNING, I'D PROBABLY FEEL VERY
13 DIFFERENTLY. BUT WE'RE KIND OF AT THE END, AND I'M
14 JUST NOT SURE QUITE HOW SUDDENLY CHANGING COURSE IS
15 GOING TO IMPACT ON HOW CIRM IS PERCEIVED AND VIEWED
16 AND EVALUATED BY THE PUBLIC. THANK YOU. I DO WANT
17 TO COME BACK TO THE VOTING PROCESS. THANK YOU.

18 CHAIRMAN SHEEHY: I DID HAVE SOME
19 QUESTIONS ABOUT THE PROCESS MYSELF. I GUESS FOR ME
20 I'VE ALWAYS SEEN THIS FIELD AS EVOLVING. IF YOU
21 WERE TO REALLY THINK ABOUT HOW THE AGENCY WAS
22 ESTABLISHED, WE WOULD BE RESTRICTING OURSELVES
23 ALMOST EXCLUSIVELY TO EMBRYONIC STEM CELL RESEARCH.

24 SO I GUESS IN TERMS OF THE CONTEXT --
25 WE'VE ALWAYS KIND OF FOLLOWED THE SCIENCE. AND SO I

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1 AM A BIT RELIANT ON SOME OF THE SCIENTIST MEMBERS OF
2 THE ICOC AND SOME OF THE INPUT FROM THE CIRM TEAM
3 THAT THIS IS WHERE THE SCIENCE IS GOING. AND IT
4 DOES SEEM LIKE -- I GUESS THERE'S A LITTLE NUANCE
5 HERE IN THAT DO I THINK NECESSARILY THIS SHOULD BE A
6 MAJOR FOCUS FOR CIRM? NOT PARTICULARLY. I KIND OF
7 LIKE THE WAY IN WHICH THEY NARROWED IT TO UNMET
8 MEDICAL NEED UNLIKELY TO FIND FUNDING FROM OTHER
9 SOURCES, BUT IT DOES SEEM LIKE, FOR SOME OF THE WORK
10 THAT WE'VE DONE, THE NEXT STEP IN THE PROGRESSION
11 TOWARDS SCALABLE, EFFECTIVE TREATMENTS. AND SO IT
12 SEEMS LOGICAL TO ME.

13 AND THE THING ABOUT SMALL MOLECULES AND
14 ANTIBODIES IS THAT THOSE ARE SO FUNDABLE BY OTHER
15 SOURCES. THAT'S WHY I PERSONALLY WAS MORE
16 SUPPORTIVE OF NARROWING THAT, BUT IF WE HAVE UNMET
17 MEDICAL NEEDS AND WE HAVE PROJECTS THAT ARE REVIEWED
18 FAVORABLY BY OUR SCIENTIFIC REVIEWERS, AND THEY'RE
19 UNLIKELY TO GET FUNDING FROM OTHER SOURCES, IT SEEMS
20 LIKE THAT FITS IN LINE WITH SOME OF THE WORK WE'VE
21 DONE. AND I JUST THINK ABOUT, WHETHER IT'S THE WORK
22 WE'VE DONE IN SKID, THE WORK WE HOPE TO DO ON SICKLE
23 CELL DISEASE, IF THE NEXT PHASE AFTER TAKING CELLS
24 OUT, MODIFYING THEM, AND PUTTING THEM BACK IS SOME
25 SORT OF PRODUCT THAT WORKS ON THE CELLS TO MODIFY

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1 THEM, IT SEEMS OVERLY RESTRICTIVE NOT TO HAVE THAT
2 ABILITY TO FUND THAT, I GUESS, IS THE POINT THE WAY
3 I KIND OF VIEW IT.

4 DR. DEAS: AND I WOULD AGREE WITH THAT.
5 WHILE I THINK THIS IS VERY IMPORTANT, I THINK BACK
6 AT THE LAST MEETING THAT WE HAD WHEN WE WERE KIND OF
7 RATIONING THE LITTLE BIT OF MONEY THAT WE HAVE LEFT
8 AND ALL THE THINGS THAT WE PUT IN CERTAIN
9 COMPARTMENTS WHERE THE FUNDING WOULD COME FROM. AND
10 I'M PARTICULARLY CONCERNED ABOUT THE SICKLE CELL
11 RESEARCH. I CERTAINLY DON'T WANT TO TAKE ANY
12 FUNDING AWAY FROM THAT.

13 CHAIRMAN THOMAS: I AGREE, DEBORAH. I
14 DON'T THINK THAT'S ON THE TABLE. I WANTED TO SAY I
15 SIDE WITH JEFF AND HIS COMMENTS ON THIS. IT SEEMS
16 TO ME THAT IF YOU HAVE AN OPPORTUNITY TO FUND
17 SOMETHING THAT PROPERLY FALLS WITHIN THE
18 REGENERATIVE MEDICINE DEFINITION, AND EVEN THOUGH IT
19 EXPANDS A BIT THE SCOPE OF WHAT WE'VE BEEN ABLE TO
20 DO, IF IT IS SOMETHING THAT IS NOT GOING TO BE
21 FUNDED ELSEWHERE AND CAN ADVANCE THE BALL AND IS
22 CUTTING EDGE AND ALL OF THAT, THAT IT MAKES SENSE TO
23 ME TO INCLUDE THIS AS SOMETHING THAT CAN GET FUNDED
24 UNDER THIS -- UNDER OUR GUIDELINES. SO I WOULD BE
25 SUPPORTIVE OF THE RECOMMENDATION HERE.

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1 DR. JUELSGAARD: DR. SAMBRANO, MR. THOMAS
2 JUST MADE A COMMENT, AND I WANT SOME CLARIFICATION.
3 HE INDICATED THAT HE THOUGHT THAT WHAT WE WERE
4 TALKING ABOUT IN TERMS OF ADDING WAS WITHIN THE
5 SCOPE OF THE BROAD TERM OF REGENERATIVE MEDICINE.
6 MY UNDERSTANDING ACTUALLY IS NOT -- IT'S THAT IT'S
7 NOT. THIS IS A REGENERATIVE MEDICINE PLUS SOMETHING
8 OUTSIDE OF THE AREA OF REGENERATIVE MEDICINE; IS
9 THAT CORRECT, OR IS THIS WITHIN THE REGENERATIVE
10 MEDICINE FIELD?

11 DR. SAMBRANO: IT'S A GOOD QUESTION. IT
12 IS CONSIDERED TO BE WITHIN REGENERATIVE MEDICINE
13 ACTUALLY BY MANY GROUPS INCLUDING THE FDA. EVEN WE
14 ARE VERY SPECIFICALLY TARGETING GENE THERAPY
15 APPROACHES, AND THAT'S WHY WE INCLUDED IN THE
16 PROJECT ELIGIBILITY DEFINITION THAT IT IS INTENDED
17 TO REPLACE, REGENERATE, REPAIR A FUNCTION OF AGED,
18 DISEASE DAMAGED, OR DEFECTIVE CELLS, TISSUES, AND
19 ORGANS, WHICH ESSENTIALLY IS THE DEFINITION OF
20 REGENERATIVE MEDICINE. SO THE INTENT IS GENE
21 THERAPY IN THE SERVICE OF REGENERATIVE MEDICINE.

22 DR. STEWARD: I'M SORRY TO INTERRUPT YOU.
23 JUST TO SAY AND EXPAND JUST A TINY BIT ON GIL'S
24 COMMENTS. ACTUALLY I WOULD SAY, AND I THINK THE FDA
25 WOULD PROBABLY AGREE, THAT REGENERATIVE MEDICINE

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1 ACTUALLY GOES EVEN FURTHER THAN THAT. AND THE
2 DEFINITION OF REGENERATIVE MEDICINE THAT GIL GAVE IS
3 SPOT ON. IT CERTAINLY INCLUDES STEM CELLS. IT
4 CERTAINLY INCLUDES GENE THERAPY AS WE'VE DEFINED IT.
5 IT ALSO INCLUDES OTHER ASPECTS OF MODIFICATION OF
6 GENES THAT ARE MEANT TO TREAT A DISEASE OR DISORDER.
7 SO SOME OTHER THINGS TOO, INCLUDING SMALL MOLECULES.

8 SO I THINK THAT ACTUALLY WE HAVE CONFLATED
9 THE TERM WITH STEM CELLS, BUT THE REST OF THE WORLD
10 REALLY DOESN'T.

11 AND I'LL JUST SAY, AS LONG AS I'M TALKING,
12 IF WE WERE SITTING HERE NOW AND REDEFINING THE SCOPE
13 OF WHAT CIRM 3.0 MIGHT LOOK LIKE, SHOULD WE BE
14 FORTUNATE ENOUGH TO BE REFUNDED, I THINK THAT'S THE
15 DISCUSSION WE NEED TO HAVE. WHAT REALLY IS THE
16 SCOPE OF REGENERATIVE MEDICINE TODAY IN THE CURRENT
17 SCIENTIFIC LANDSCAPE? IT WOULD INCLUDE WHAT WE'RE
18 TALKING ABOUT, BUT IT WOULD INCLUDE MORE AS WELL.

19 SO PART OF THE REASON THAT I'M JUST A
20 LITTLE TINY BIT UNCOMFORTABLE ABOUT SORT OF THE
21 ONE-OFF, I'M FINE WITH IT, BUT IT ABSOLUTELY DOES
22 IMPACT ON THE AMOUNT OF MONEY WE HAVE AVAILABLE FOR
23 STEM CELLS BECAUSE THIS IS A ZERO-SUM GAME AT THIS
24 POINT. WE SPEND ON ONE; WE CAN'T SPEND ON ANOTHER.
25 THANK YOU. I'LL STOP. I DO WANT TO GET BACK TO

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1 PROCESS THOUGH.

2 CHAIRMAN SHEEHY: I DO TOO.

3 I WOULDN'T MIND RESPONDING TO THAT, BUT
4 ARE THERE OTHER FOLKS WHO WANT TO HAVE COMMENTS
5 BECAUSE I DON'T WANT TO MONOPOLIZE?

6 WHAT I DID FIND INTERESTING ABOUT OS'
7 COMMENTS WAS THAT IT ACTUALLY DID RAISE A QUESTION
8 OF WHAT WILL BE THE SCOPE OF CIRM 3.0 WHICH IS POST
9 A POSSIBLE ELECTION, A BOND MEASURE. IT'S
10 INTERESTING THAT THIS IS THE FIRST TIME THAT WE'VE
11 ACTUALLY HAD THAT DISCUSSION, THAT WE ARE LARGELY
12 NOT -- DOESN'T SEEM LIKE WE'RE GOING TO BE PART OF
13 THAT DISCUSSION AT ALL. SO THAT'S ONE POINT THAT I
14 WAS STRUCK BY.

15 AND SO MAYBE THAT IS SOMETHING THAT WE
16 WANT TO TAKE INTO ACCOUNT BECAUSE WE HAVE NO
17 INFLUENCE OVER THE CONSTRUCTION OF THAT MEASURE.
18 AND SO TO THE DEGREE THAT WE THINK THAT THE
19 DEFINITION OF REGENERATIVE MEDICINE NEEDS TO BE
20 EXPANDED, PERHAPS THIS MAKES IT MORE COMPELLING THAT
21 WE TAKE THIS STEP BECAUSE WE HAVE NO OTHER WAY TO
22 PUT A MARKER ON THE TABLE. WE ARE NOT INVOLVED IN
23 THE DISCUSSION OF THE CONSTRUCTION OF THE NEXT
24 BALLOT MEASURE. WHATEVER WE LAUNCH NOW WILL HAVE AN
25 INFLUENCE ON WHAT HAPPENS IN THAT FUTURE THAT WE

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1 CAN'T CONTROL OTHER THAN WHAT WE DO NOW WITHIN OUR
2 CURRENT CONSTRUCT.

3 AND THE SECOND POINT, I GET THE ZERO-SUM
4 GAME ASPECT OF THIS, BUT TO ME THERE REALLY IS NO
5 SUCH THING AS A ZERO-SUM GAME IN THIS FIELD. IN
6 THIS, IF WE ARE MEETING THE DEFINITIONS, WHICH ARE
7 UNMET MEDICAL NEED, I THINK THE WAY IN WHICH, AND
8 DR. SAMBRANO ARTICULATED THE KIND OF REGENERATIVE
9 MEDICINE DEFINITION THAT'S INCLUDED IN THIS
10 PROPOSAL, THESE ARE CURATIVE. ONE OF THE MOST
11 EXCITING THINGS ABOUT REGENERATIVE MEDICINE IS WE'RE
12 ACTUALLY TRYING TO CURE DISEASE. AND BEING FROM THE
13 HIV FIELD AND TAKING EXPENSIVE MEDICATIONS EVERY DAY
14 OR ELSE I DIE, THE CHRONIC DISEASE MODEL I JUST
15 DON'T THINK IS SUSTAINABLE EITHER FOR INDIVIDUALS OR
16 FOR SOCIETY.

17 AND SO IF WE DID GET SOMETHING THAT CAME
18 THROUGH THAT WAS RATED EXCEPTIONAL, AS OUR
19 APPLICATIONS HAVE TO BE TO GET FUNDED, THAT MET AN
20 UNMET MEDICAL NEED, THE SCALABILITY THAT IS INHERENT
21 IN THIS APPROACH MAKES IT EXTREMELY ATTRACTIVE. SO
22 I WOULDN'T SEE THAT AS A ZERO-SUM GAME. I WOULD SEE
23 THAT AS A VICTORY. BUT HAPPY -- ANY OTHER THOUGHTS
24 ARE WELCOME.

25 DR. STEWARD: JUST A COMMENT TO JEFF. I

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1 THINK YOU'RE SPOT ON ABOUT ALL OF YOUR COMMENTS
2 ABOUT, I'M GOING TO CALL IT CIRM 3.0 JUST FOR
3 CONVENIENCE, THAT WE ARE NOT INVOLVED, BUT I THINK
4 THAT IN THE CONSTRUCTION OF WHATEVER IS GOING
5 FORWARD, THERE CERTAINLY WILL BE A LOOK BACK AT WHAT
6 THE SCOPE CURRENTLY IS. SO WHAT WE'RE DOING TODAY
7 ACTUALLY DOES HAVE, I THINK, SOME VERY SIGNIFICANT
8 POSITIVE IMPACT. I USE THE WORD "POSITIVE" NOT IN
9 THE EVALUATIVE SENSE, BUT JUST IN TERMS OF WHAT IT
10 WILL LIKELY DO GOING FORWARD.

11 EVEN IF THE THE MEASURE DOESN'T HAVE THE
12 SAME DEFINITION, I THINK MOST OF THE PUBLIC WILL BE
13 CONFUSED ABOUT WHAT IS AND WHAT IS TO BE, JUST TO
14 SAY. I WOULD HOPE ACTUALLY THAT WHATEVER GOES
15 FORWARD WOULD BE SOMEWHAT REFLECTIVE OF WHAT WE'VE
16 BEEN DOING.

17 JUST A COMMENT ON THE ZERO-SUM GAME. I'M
18 JUST TALKING ABOUT MONEY. WE HAVE X AMOUNT LEFT.
19 SAY THAT MONEY WISER, THAT'S WHAT'S LEFT. THAT'S
20 ALL I MEANT ABOUT THAT. THANK YOU.

21 CHAIRMAN SHEEHY: WELL, I WONDER IF OUR
22 NEXT STEP MIGHT BE TO KIND OF TAKE THIS IN TWO PARTS
23 AND VOTE ON THE OVERALL CONCEPT, AND THEN HAVE A
24 SECOND DISCUSSION AND VOTE ON THE PROCESS. IS THAT
25 SOMETHING THAT PEOPLE WOULD BE COMFORTABLE WITH?

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1 AND IF SO, CAN I GET A MOTION SO AT LEAST WE CAN
2 DRAW TOWARDS SOME SORT OF DECISION POINT?

3 CHAIRMAN THOMAS: SO MOVED.

4 CHAIRMAN SHEEHY: THE MOTION, AS I TAKE
5 IT, IS TO ACCEPT THE CONTEXT, AND THEN WE'LL HAVE
6 ANOTHER DISCUSSION ABOUT THE PROCESS. IS THERE A
7 SECOND ON THAT?

8 DR. DULIEGE: I SECOND.

9 CHAIRMAN SHEEHY: GREAT. MARIA, COULD YOU
10 CALL THE ROLL PLEASE.

11 MR. TOCHER: I JUST WANTED TO CLARIFY THAT
12 THIS IS APPLICABLE TO BOTH CONCEPT PLAN CHANGES FOR
13 THE TRAN AND DISC PROGRAMS -- CLIN.

14 CHAIRMAN SHEEHY: I THINK, JUST TO BE
15 CLEAR, I THINK IT'S SLIDE 2, 3, 4. AND THEN THE
16 NEXT THING WE WILL DISCUSS WILL BE SLIDE 5.

17 MS. BONNEVILLE: DEBORAH DEAS.

18 DR. DEAS: YES.

19 MS. BONNEVILLE: ANNE-MARIE DULIEGE.

20 DR. DULIEGE: YES.

21 MS. BONNEVILLE: DAVID HIGGINS.

22 DR. HIGGINS: YES.

23 MS. BONNEVILLE: STEVE JUELGAARD.

24 MR. JUELGAARD: YES.

25 MS. BONNEVILLE: JEFF SHEEHY.

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1 CHAIRMAN SHEEHY: YES.
2 MS. BONNEVILLE: OS STEWARD.
3 DR. STEWARD: YES.
4 MS. BONNEVILLE: JONATHAN THOMAS.
5 CHAIRMAN THOMAS: YES.
6 MS. BONNEVILLE: KRISTINA VUORI.
7 DR. VUORI: YES.
8 MS. BONNEVILLE: MOTION CARRIES.
9 CHAIRMAN SHEEHY: GREAT. THANK YOU. AND,
10 OS, YOU HAD SOME THOUGHTS ABOUT THE PROCESS?
11 DR. STEWARD: YES.
12 CHAIRMAN SHEEHY: GO AHEAD PLEASE.
13 CHAIRMAN THOMAS: I THINK THAT WAS A
14 VOTING YES. I'M NOT SURE HE HEARD YOUR QUESTION.
15 CHAIRMAN SHEEHY: YOU HAVE THOUGHTS ON THE
16 PROCESS?
17 DR. STEWARD: YEAH. SO AS I UNDERSTAND
18 IT, THE PROVISION IN PROP 71 ABOUT VITAL RESEARCH --
19 I ALWAYS FORGET WHAT IT IS -- VITAL RESEARCH NEED --
20 CHAIRMAN SHEEHY: OPPORTUNITY.
21 DR. STEWARD: I THINK IT WAS A SIGNAL THAT
22 WE NEED TO CONSIDER THIS TO BE SOMETHING
23 EXCEPTIONAL. AND IN THAT REGARD, I'M CONCERNED
24 ABOUT THE BLANKET TWO-THIRDS VOTE FOR AN AREA OF
25 RESEARCH. AN AREA OF RESEARCH IS ONE THING, BUT

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1 REALLY WHAT I THINK NEEDS TO BE DONE IS THAT THIS
2 VITALITY OF IT, IF YOU WILL, REALLY HAS TO BE
3 DETERMINED ON A PROJECT-BY-PROJECT BASIS.

4 SO I'M FINE WITH CONCEPTS FALLING UNDER
5 THIS GOING FORWARD, BUT I'M NOT FINE WITH A FAILURE
6 TO HAVE A VOTE BY THE GWG ON EACH AND EVERY PROJECT.
7 THANK YOU.

8 CHAIRMAN SHEEHY: I FEEL LIKE YOU READ MY
9 MIND. I HAD THE SAME THOUGHT WHEN I SAW THAT, THAT
10 IT NEEDED TO BE PER PROJECT THAT THE VOTE BE TAKEN.
11 BUT I THINK WE ARE ALIGNED, BUT ARE THERE OTHER
12 THOUGHTS OR COMMENTS AMONG OTHER MEMBERS OF THE
13 BOARD OR CIRM TEAM ON THAT?

14 DR. SAMBRANO: I CAN TELL YOU THE
15 RATIONALE FOR WHY WE WERE DOING IT IS TO SIMPLIFY
16 THE PROCESS. OUR THOUGHTS WERE THAT IF THE BOARD
17 AND THE GWG CONSIDER THIS ARENA TO BE VITAL ENOUGH
18 TO INCLUDE WITH OUR STEM CELL CORE PROJECTS, THAT
19 THAT MAY BE ENOUGH.

20 I THINK ONE OF THE OTHER THINGS THAT WE
21 THOUGHT MIGHT BE DIFFICULT IS TO TRY TO DO THIS AT
22 THE BEGINNING. I THINK THERE WAS A SUGGESTION TO
23 TRY TO HAVE A PROCESS THAT ALLOWS US AT THE ONSET TO
24 HAVE THE GWG TAKE A VOTE ON PROJECTS. BUT I THINK
25 THE GWG WON'T REALLY BE ABLE TO COME TO A CONCLUSION

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1 ON WHETHER THEY AGREE THAT THIS IS A VITAL RESEARCH
2 OPPORTUNITY UNTIL THEY DO A FULL REVIEW, WHICH MEANS
3 THAT A PROJECT CAN GO THROUGH THE FULL PROCESS OF
4 APPLICATION AND REVIEW ONLY TO FAIL AT THE POINT
5 WHERE THE VOTE DOES NOT ALLOW THEM TO MOVE FORWARD,
6 WHICH IS FINE. WE CAN DO IT THAT WAY. IT WAS JUST
7 INTENDED TO TRY TO CAPTURE THIS ARENA AS A WHOLE AND
8 DETERMINE THAT THIS IS SOMETHING THAT IS IMPORTANT.

9 DR. STEWARD: I DON'T THINK AT THIS POINT
10 I'D BE WORRIED ABOUT SIMPLIFYING THE PROCESS. I
11 THINK WHAT WE NEED TO DO IS GET IT RIGHT. AND I
12 WOULD BE PERFECTLY FINE WITH HAVING A FULL REVIEW ON
13 IT AND THEN A VOTE BECAUSE IT REALLY IS SOMETHING
14 THAT IT'S NOT IN THE SAME BASKET AS THE REST OF OUR
15 STEM CELL GRANTS. IT SHOULD BE, AS I UNDERSTAND IT,
16 THE PROVISION IN PROP 71. SO I ACTUALLY THINK THERE
17 SHOULD BE A FULL REVIEW OF THE PROJECT. THANK YOU.

18 CHAIRMAN SHEEHY: I AGREE WITH YOU, OS. I
19 JUST THINK ABOUT -- HOW MANY DISCUSSIONS HAVE WE HAD
20 ABOUT THE HUNT FOR THE ILLUSIVE CANCER STEM CELL OR
21 CANCER-INITIATING CELL? EVEN THOUGH THAT'S PREBAKED
22 BY INCLUSION WITHIN THE SCOPE, I THINK SOMETIMES
23 PROJECTS KIND OF FALL OFF THE MAP BECAUSE THE LINK
24 IS CONTINUOUS BUT ALSO, BECAUSE IN A KIND OF
25 IMPLICIT REJECTION OF IT BEING A VITAL RESEARCH

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1 OPPORTUNITY, PEOPLE TEND NOT TO BE THAT SUPPORTIVE
2 OF SOME OF THESE PROJECTS. THAT'S BEEN KIND OF, AT
3 LEAST TO ME, ALMOST LIKE AN UNDERLYING THING. IT'S
4 LIKE ANYBODY CAN FUND THIS. WE DON'T REALLY SEE A
5 CANCER STEM CELL. AND THEN WE HAVE KIND OF THIS
6 DISCUSSION, BUT IT'S IN SCOPE, SO IT GETS REALLY
7 MUDDY. I THINK THIS ACTUALLY FOR THIS KIND OF
8 OPPORTUNITY WOULD ACTUALLY BRING MORE CLARITY AND
9 THAT PEOPLE CAN ACTUALLY HAVE A VOTE ON THAT WHEN
10 THEY HAVE QUESTIONS ABOUT THE RELEVANCE TO CIRM'S
11 PROGRAMS.

12 DO WE HAVE ANY OTHER DISCUSSIONS? DO YOU
13 WANT TO MAKE A MOTION, OS, BUT I'M HAPPY IF OTHER
14 PEOPLE HAVE COMMENTS THEY WANT TO MAKE.

15 MR. TOCHER: JUST ON A PROCESS POINT. WE
16 ARE HAPPY TO GET A SENSE OF THE COMMITTEE AND
17 HOWEVER YOU WISH TO PROCEED FOR THE PROCESS PART.
18 IT'S NOT SOMETHING CIRM WOULD FEEL LIKE WE NEED A
19 MOTION IN ORDER FOR US TO IMPLEMENT IT IF THAT'S THE
20 WISH OF THE COMMITTEE.

21 CHAIRMAN SHEEHY: I THINK MAYBE TO DISTILL
22 IT JUST FOR ABSOLUTE CLARITY, AND I'M GOING TO
23 PARAPHRASE YOU, OS, THAT IT REALLY MIRROR THE
24 LANGUAGE IN PROP 71. AND AFTER THE VOTE IS TAKEN,
25 THEN A SECOND VOTE IS REQUIRED OF THE GWG TO DEEM

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1 THIS A VITAL RESEARCH OPPORTUNITY. I THINK THAT'S
2 WHAT YOU HAD INTENDED, OS. I THINK THAT'S FAIRLY
3 CLOSE TO THE LANGUAGE IN PROP 71.

4 DR. STEWARD: THAT'S PERFECT, JEFF. THANK
5 YOU.

6 CHAIRMAN SHEEHY: SO IF THERE'S NOT THE
7 FEELING THAT WE NEED A MOTION, THAT'S FINE. WILL
8 THAT BE INCLUDED IN THE PROPOSAL THAT'S BROUGHT TO
9 THE BOARD FOR THE FULL VOTE?

10 DR. SAMBRANO: YES.

11 CHAIRMAN SHEEHY: GREAT. DO WE HAVE ANY
12 PUBLIC COMMENT ON ANY OF THESE ISSUES?

13 MR. TOCHER: NOT HERE AT CIRM.

14 CHAIRMAN SHEEHY: DO WE HAVE ANY OTHER
15 DISCUSSION? THEN IF EVERYBODY IS FINE, I'M HAPPY TO
16 ADJOURN THE MEETING. AND THANK YOU, EVERYONE, FOR
17 HOPPING IN FOR THIS. I KNOW IT'S SOMETIMES
18 DIFFICULT TO DO, BUT I GREATLY APPRECIATE IT. SO
19 WITH THAT, THE MEETING IS ADJOURNED.

20 DR. STEWARD: THANKS TO CIRM'S STAFF FOR
21 PUTTING THIS TOGETHER.

22 MS. BONNEVILLE: NEXT WEEK WE HAVE A FULL
23 TELEPHONIC BOARD MEETING. DON'T FORGET.

24 (THE MEETING WAS THEN CONCLUDED AT
25 10:41 A.M.)

REPORTER'S CERTIFICATE

I, BETH C. DRAIN, A CERTIFIED SHORTHAND REPORTER IN AND FOR THE STATE OF CALIFORNIA, HEREBY CERTIFY THAT THE FOREGOING TRANSCRIPT OF THE TELEPHONIC PROCEEDINGS BEFORE THE SCIENCE SUBCOMMITTEE OF THE INDEPENDENT CITIZEN'S OVERSIGHT COMMITTEE OF THE CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE IN THE MATTER OF ITS REGULAR MEETING HELD ON NOVEMBER 13, 2018, WAS HELD AS HEREIN APPEARS AND THAT THIS IS THE ORIGINAL TRANSCRIPT THEREOF AND THAT THE STATEMENTS THAT APPEAR IN THIS TRANSCRIPT WERE REPORTED STENOGRAPHICALLY BY ME AND TRANSCRIBED BY ME. I ALSO CERTIFY THAT THIS TRANSCRIPT IS A TRUE AND ACCURATE RECORD OF THE PROCEEDING.

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